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1: Ann N Y Acad Sci. 1998 Jun 30;850:163-77.

Targeted integration of a recombinant globin gene adeno-associated viral vector into human chromosome 19.

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Transfer of a globin gene into stem cells along with the regulatory elements required to achieve high level expression in maturing erythroid cells would provide effective gene therapy for Cooley's Anemia. We have explored the use of recombinant adeno-associated viral (rAAV) vectors for this purpose. A vector designated rHS32A gamma*3'RE that contains regulatory elements from the locus control and flanking regions, integrates as a stable head-to-tail concatamer in erythroleukemia cells at a high multiplicity of infection and exhibits high level, regulated gamma globin gene expression. Inducible expression of the non-structural Rep proteins of wild-type AAV in HeLa cells transduced with rAAV vectors does not increase overall integration frequency, but targeted integration of rHS32A gamma*3'RE into human chromosome 19 was documented.

PMID: 9668538 [PubMed - indexed for MEDLINE]

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Position-independent huma beta-globin gene expressior mediated by a recombinant adeno-associated virus vector carrying the chicken beta-globin insulator. [J Virol. 1999]

Recombinant adeno-associated virus (rAAV)-mediated expression of a human gamma-globin gene in human progenitor-derived erythroid cells. [Proc Natl Acad Sci U S A. 1994]

Cellular recombination pathways and viral terminal repeat hairpin structures ar sufficient for adeno-associated virus integration in vivo and in vitro. [J Virol. 1997]

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